

Stories of Hope: Leukemia

Español

Exposing Secrets, Saving Lives



Stem cells create life. But if things go wrong, they can also threaten it. **Theresa Blanda** found that out the hard way. Fortunately for her, CIRM-funded research helped her fight this threat, and get her life back.

In the first few days of human development embryonic stem cells are a blank slate; they don't yet have a special, defined role, but have potential. The potential to turn into the cells that make up our kidneys, heart, brain, every other organ and every tissue in our body. Because of this flexibility, stem cells have shown great promise as a way to regenerate dead, diseased or injured tissue to treat many life-threatening or chronic conditions.

But some studies have suggested a secret, darker side to stem cells—so-called cancer stem cells. Like their embryonic cousins, these cells have the ability to both self-renew— to divide and make more copies of themselves - and specialize into other cell types. Many researchers believe they can serve as a reservoir for cancer, constantly reinvigorating tumors, helping them spread throughout the body. To complicate matters, these slow-growing cells are often impervious to cancer therapies, enabling them to survive chemotherapy.

For Theresa Blanda, cancer stem cells were dragging her down a slippery slope towards disease and possibly death. In 2003, she was diagnosed with polycythemia vera (PV), which causes the body to produce too many red blood cells. As sometimes happens with PV patients, her body began producing too many white blood cells as well. Eventually, she developed an even more serious condition, myelofibrosis, a form of bone marrow scarring that results in an enlarged spleen, bone pain, knee swelling and other debilitating symptoms.

"You couldn't even breathe my way or I'd bruise," says Theresa. "I didn't think I was going to make it."

Her doctors wanted to do a bone marrow transplant, but were having difficulty finding the right donor. "Finally, I just asked if there was some kind of clinical trial that could help me," says Theresa.

Fortunately, there was.

The Root Cause

At UC San Diego's Moores Cancer Center, Catriona Jamieson, M.D., Ph.D., had made a discovery that would have a big impact on Theresa's health. In research funded in part by CIRM, Jamieson found a key mutation in blood-forming stem cells. Specifically, a mutation in a gene called JAK2 was being passed on to Theresa's entire blood system, causing PV and myelofibrosis. Without effective

treatment, her condition could have progressed into acute myeloid leukemia, a blood cancer with a very poor survival rate.

"These malignant stem cells create an inhospitable environment for regular stem cells, suppressing normal blood formation," says Jamieson. "We needed to get rid of these mutated stem cells so the normal ones could breathe a sigh of relief."

The answer was a JAK2 inhibitor being developed by San Diego-based TargeGen. Though the trial had already started, they made room for Theresa and the results were amazing. Within weeks, her discomfort had faded, her spleen had returned to normal and she was back at work.

"In a month or two I was feeling pretty good," says Theresa. "I could climb stairs and the swelling in my knee had gone down."

She continued on the drug for five years but safety issues forced the trial to be suspended. But the work continues. With continued support from CIRM, Jamieson and others are investigating new JAK2 inhibitors, and other alternatives, to help myelofibrosis patients.

"Because of CIRM funding, we've managed to develop a number of agents that have gone into clinical trials," says Jamieson. "That means patients have lived to hold their grandchildren, attend their mom's hundredth birthday party and live fruitful lives."

For more information about CIRM-funded leukemia research, visit our fact sheet.

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